



Solid Biosciences Highlights Expertise in AAV Gene Therapy with Multiple Abstracts to be Presented at the American Society of Gene & Cell Therapy 24th Annual Meeting

May 4, 2021

-Data from studies of novel AAV vectors and AAV immunology and an encore of 12-month safety, efficacy and patient-reported outcomes measurements from the ongoing IGNITE DMD trial of SGT001 to be presented-

CAMBRIDGE, Mass., May 04, 2021 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today announced the presentation of three abstracts at the upcoming American Society of Gene & Cell Therapy (ASGCT) 24th Annual Meeting. The abstracts report data from research-stage studies of novel adeno-associated viral (AAV) vectors for muscle gene delivery and mechanisms of immune responses to AAV9, as well as 12-month data from the ongoing IGNITE DMD trial Phase I/II study of SGT-001 microdystrophin gene therapy in patients with Duchenne, which were previously presented at the Muscular Dystrophy Association Virtual Clinical & Scientific Conference in [March 2021](#). The ASGCT Annual Meeting is being held virtually May 11-14, 2021.

Presentation Details

Title: AAV9 Capsid-Anti-AAV9 Antibody Immune Complexes Promote Complement Activation and Cytokine Release In Vitro

Presenter: Qian Chen, Senior Director, R&D, Solid Biosciences

Presentation Type: Digital poster

Abstract Number: 747

Title: Continued In Vitro and In Vivo Characterization of Novel AAV Vectors Engineered for Muscle Gene Delivery

Presenter: Jennifer Green, PhD, Principal Scientist, R&D, Solid Biosciences

Presentation Type: Digital poster

Abstract Number: 319

Title: IGNITE-DMD: Phase I/II Study of Single SGT-001 Microdystrophin Gene Therapy for DMD

Presenter: Carl Morris, PhD, Chief Scientific Officer, Solid Biosciences

Presentation Date & Time: Friday, May 14, 2021, 1:45-2:00 PM ET

Presentation Type: Oral

Abstract Number: 263

Abstracts for the presentations can be viewed online at: <https://annualmeeting.asgct.org/>

About SGT-001

Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne. Duchenne is caused by mutations in the dystrophin gene that result in the absence or near absence of dystrophin protein. SGT-001 is a systemically administered candidate that delivers a synthetic dystrophin gene, called microdystrophin, to the body. This microdystrophin encodes for a functional protein surrogate that is expressed in muscles and stabilizes essential associated proteins, including neuronal nitric oxide synthase (nNOS). Data from Solid's clinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, regardless of genetic mutation or disease stage.

SGT-001 is based on pioneering research in dystrophin biology by Dr. Jeffrey Chamberlain of the University of Washington and Dr. Dongsheng Duan of the University of Missouri. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and European Union.

About Solid Biosciences

Solid Biosciences is a life sciences company focused on advancing transformative treatments to improve the lives of patients living with Duchenne. Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001. For more information, please visit www.solidbio.com.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the ability of the Company to continue dosing patients in the IGNITE DMD trial, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 in patients with DMD, the Company's expectations for reporting future data from the IGNITE DMD trial, the Company's regulatory plans and timelines and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," "working" and similar expressions. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the Company's ability to or continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approvals from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll patients in IGNITE DMD on the timeline expected; the Company's dosing strategy; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; whether the interim data presented in this release will be predicative of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001; whether the methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters

will yield meaningful statistical results; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the Company's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views as of the date hereof and should not be relied upon as representing the Company's views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

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